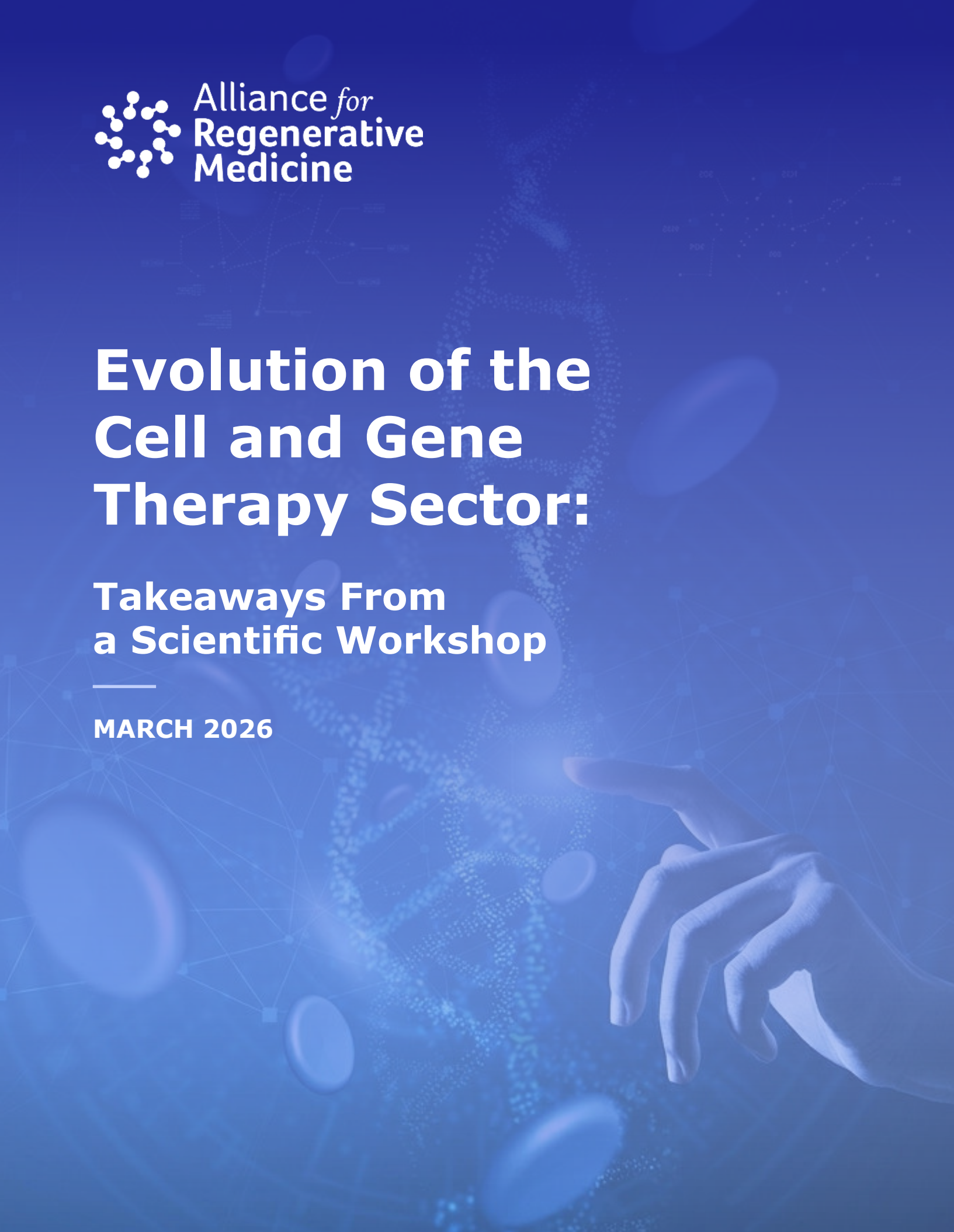
Alliance for Regenerative Medicine

Evolution of the Cell and Gene Therapy Sector:

Takeaways From a Scientific Workshop

MARCH 2026



1 INTRODUCTION

From November 5th to 6th, 2025, the **Alliance for Regenerative Medicine (ARM)** hosted a 2-day workshop to explore the next phase of the CGT sector. Bringing together developers, clinicians, patient advocates, and other key stakeholders from across the CGT industry (see the Appendix for a full list of presenters and panelists), this workshop represented ARM's continued commitment to helping the global CGT sector achieve modernization in healthcare systems, overcome obstacles to innovation, and increase patient access to life-changing treatments. The workshop extended momentum from a UK-based workshop on CGT manufacturing and industrialization in the spring of 2025 (*see whitepaper from that event here*).

The goals of the November workshop were to explore the scientific, regulatory, and patient-access challenges shaping the CGT field, with a focus on scaling advanced therapies to reach more patients worldwide. Case studies[1] and panel discussions highlighted (1) scale-in, scale-out, and scale-up strategies for advancing autologous and allogeneic CGTs toward industrialization; (2) innovations in viral and nonviral delivery; (3) advances in analytics to accelerate drug release; (4) strategies for improving patient participation in, and community access to, CGTs; and (5) important regulatory considerations facing the CGT sector.

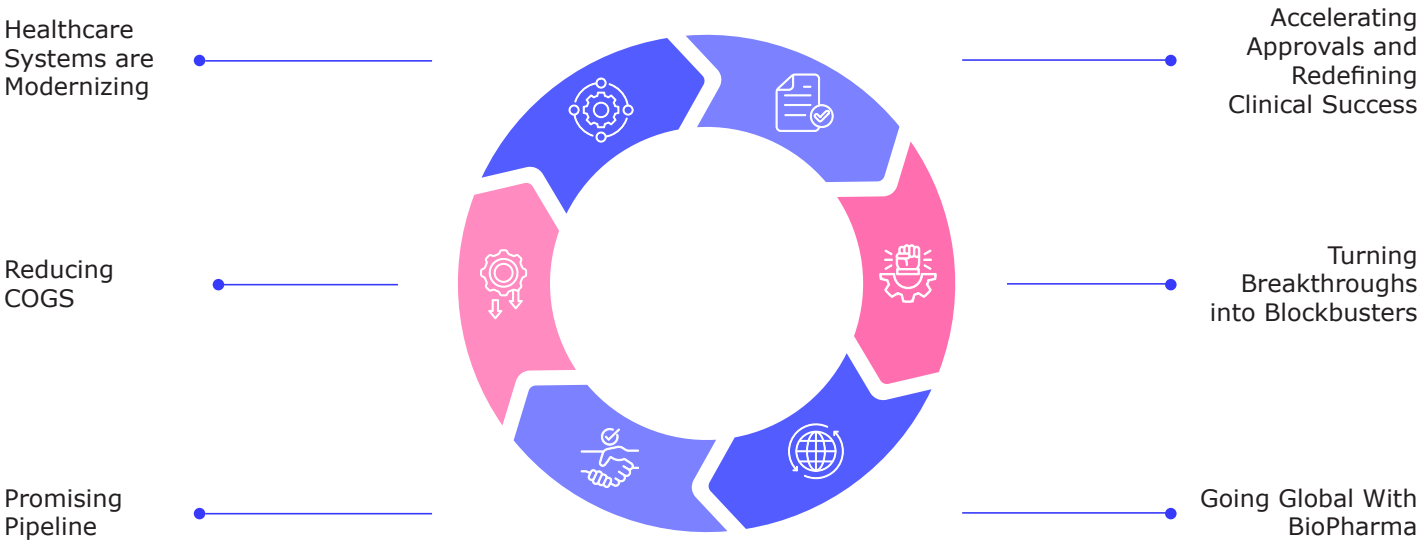
2 STATE OF THE INDUSTRY:
Undeniable acceleration and a promising outlook

The evolution of CGTs has followed a nonlinear journey, rich with great triumphs and significant setbacks. Despite continued challenges, acceleration within the field is undeniable. In the rare-disease space alone, 10 gene therapies were approved between 2023 and 2025 in the United States, compared with 5 total between 2017 and 2022. Two CAR-T products (Carvykti® and Breyanzi®) achieved "commercial blockbuster" status (defined by ARM as estimated >$1 billion in worldwide sales) in 2025, adding to other CGTs (Zolgensma® and Yescarta®) that reached "blockbuster" status between 2021 and 2024.

Current sector developments give many reasons to believe in CGTs (**Box 1**). Three key pipeline trends are driving excitement, including progress in solid tumors, CAR-T advances, and milestones with *in vivo* CGTs. Within the solid-tumor space, 2024 saw the first 2 FDA approvals for cell therapies to treat solid tumors (melanoma and synovial sarcoma), and the early-stage pipeline is strong. CAR-T advances include a move into earlier lines of treatment for multiple myeloma (with first-line testing underway), significant mid- to late-stage trials in the autoimmune-disease space (e.g., lupus and myasthenia gravis), and the 2025 decision by the FDA to drop REMS requirements and make other labeling updates to reduce logistical and cost burdens on patients and providers.[1] Two *in vivo* CRISPR gene editing therapies are in phase 3 trials, and *in vivo* CAR-Ts have seen recent large-pharma investment and early-phase clinical trial activity. In addition, significant activity is occurring within the prevalent-disease space. With a breadth of modalities under investigation, breakthroughs are anticipated in wet AMD and Parkinson's disease (treatments for both in phase 3 trials) as well as in multiple sclerosis, type 1 diabetes, and HeFH/premature CAD (therapies in earlier-stage trials).

Except where noted (e.g., publications cited), the other information presented at the workshop and included in this whitepaper has not been subject to peer review.

BOX 1 REASONS FOR A POSITIVE OUTLOOK IN THE CGT SPACE



The CGT sector remains focused on reducing COGS, with manufacturing advances boosting efficiency and optionality. Initially, each CGT developer needed to build a fully manual, bespoke, and extremely costly process-development and manufacturing function. Today, with capable CDMO options, developers can either buy established manufacturing capabilities or adopt a "hybrid" option, where they retain control of process development and small-scale manufacturing for clinical trials but then outsource larger-scale manufacturing. Looking forward to 2030, expectations to move toward full automation and reliance on platform technologies should further reduce COGS.

Healthcare systems are also modernizing to embrace CGTs. In addition to the removal of CAR-T REMS and updates to CAR-T monitoring requirements by the FDA,[1] the Centers for Medicare and Medicaid Innovation's CGT Access Model (testing outcomes-based agreements for sickle-cell disease gene therapies within Medicaid populations) launched in 2025, with 33 states, Washington D.C., and Puerto Rico participating.[2] Both moves aim to increase patient access to potentially life-saving therapies.

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